
How does gene therapy work?

Gene therapy is an experimental technique that uses genes to treat or prevent disease. In the future, this technique may allow doctors to treat a disorder by inserting a gene into a patient's cells instead of using drugs or surgery. I am testing several approaches to gene therapy, including:

1. Replacing a mutated gene that causes disease with a healthy copy of the gene.
2. Inactivating, or "knocking out," a mutated gene that is functioning improperly.
3. Introducing a new gene into the body to help fight a disease.

Although gene therapy is a promising treatment option for many diseases (including inherited disorders, some types of cancer, and certain viral infections), the technique remains risky and is still under study to make sure that it will be safe and effective. Gene therapy is currently being tested only for diseases that have no other cures.

Sometimes the whole or part of a gene is defective or missing from birth, or a gene can change or mutate during adult life. Any of these variations can disrupt how proteins are made, which can contribute to health problems or diseases. In gene therapy, I can do one of several things depending on the problem that is present. I can replace a gene that causes a medical problem with one that doesn't, add genes to help the body to fight or treat disease, or turn off genes that are causing problems. To insert new genes directly into cells, I use a vehicle called a "vector" which is genetically engineered to deliver the gene. Viruses, for example, have a natural ability to deliver genetic material into cells, and therefore, can be used as vectors. Before a virus can be used to carry therapeutic genes into human cells, however, it is modified to remove its ability to cause an infectious disease.

Gene therapy can be used to modify cells inside or outside the body. When it's done inside the body, I will inject the vector carrying the gene directly into the part of the body that has defective cells. In gene therapy that is used to modify cells outside of the body, blood, bone marrow, or another tissue can be taken from a patient, and specific types of cells can be separated out in the lab. The vector containing the desired gene is introduced into these cells. The cells are left, to multiply in the laboratory, and are then injected back into the patient, where they continue to multiply and eventually produce the desired effect.

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